MEDICARE MODERNIZATION ACT GUIDELINES -- FORMULARIES
CMS Strategy for Affordable Access to Comprehensive Drug Coverage
Guidelines for Reviewing Prescription Drug Plan Formularies and Procedures

1. Purpose of the Proposed Guidance

This paper is draft guidance on how CMS will review Medicare prescription drug benefit plans to assure that beneficiaries receive clinically appropriate medications at the lowest possible cost. Two key requirements in the Medicare Modernization Act (MMA) are to assure that drug plans provide access to medically necessary treatments for all and do not discriminate against any particular types of beneficiaries, and to encourage and support the use of approaches to drug benefit management that are proven and in widespread use in prescription drug plans today. The goal is for plans to provide high-quality cost effective drug benefits by negotiating the best possible prices and using effective drug utilization management techniques. This goal can be achieved through a CMS drug benefit review strategy that facilitates appropriate beneficiary access to all medically necessary Part D covered drugs along with plan flexibility to develop efficient benefit designs. Our draft formulary review process focuses on three areas:

1. Pharmacy and Therapeutics (P&T) committees. CMS will require P&T committees to rely on widely-used best practices, reinforced by MMA standards. Requiring these processes for the development of plan formularies that are designed to provide appropriate, up-to-date access for beneficiaries will allow CMS to avoid micromanaging plan development processes and give plans the flexibility to offer benefit designs that provide affordable access to medically necessary drugs.

2. Formulary lists. CMS' review of plan formularies will look to best practices in existing drug benefits serving millions of seniors and people with disabilities to ensure non-discriminating, appropriate access for Medicare beneficiaries. CMS will evaluate formulary classification systems as well as the actual list of drugs included in the formulary.

3. Benefit management tools. CMS will compare plans' use of benefit management tools, like prior authorization, to the way these tools are used in existing plans, to ensure that they are being applied in a clinically appropriate and non-discriminatory fashion. We also will protect beneficiary rights by putting appropriate appeals and exceptions standards in the final regulations and by reviewing processes that plans use to provide timely access. In developing this approach, CMS has looked to existing national drug benefit management standards and guidelines that underlie drug plans that are currently providing effective coverage to millions of seniors and persons with disabilities, as well as a variety of examples of such drug plans.

CMS is developing the final rule for the new Medicare drug benefit based on extensive public comments on how best to provide access to up-to-date medical treatments for all

beneficiaries at the lowest possible cost. While the policies and procedures that will be incorporated into the final rule for the Medicare outpatient drug benefit are still being refined, this paper sets forth for additional public comment the kind of "sub regulatory" approach we are considering in conjunction with the final regulation to promote transparency, predictability, and effective implementation of the law in conjunction with our final rule. CMS encourages public input on this proposed strategy; commenters need not, however, reiterate points made in their comments on the proposed regulation. Comments will be incorporated into the final guidelines, which will be released in January.

2. Background

The addition of a prescription drug benefit to Medicare as a result of the MMA represents a landmark change to the Medicare program, a change that will significantly improve the healthcare coverage available to millions of Medicare beneficiaries. For the final regulation we are considering policies, such as formulary requirements and exceptions and appeals processes, to assure that beneficiaries have access to covered drugs that are medically necessary for their condition while enabling plans to design and manage their formularies to provide the most affordable benefit possible.

The MMA is designed to encourage private sector organizations that meet the law's requirements to offer comprehensive prescription drug plan options for Medicare beneficiaries by providing flexibility for plan design and management. This flexibility is modeled after the way most Americans, including millions of seniors and people with disabilities, receive drug benefits today through federal and private-sector retiree coverage and State Medicaid programs. How much beneficiaries save depends on how a plan's formulary is structured and the benefit is operated. The goal of this program, however, is not to save money on prescription drug costs at the expense of appropriate medical care. Appropriate medical care would not be possible if plans sought to discourage enrollment by beneficiaries with high, expected drug costs.

Consequently, CMS seeks to implement a strategy to ensure that formularies and pharmacy benefit management are consistent with effective practices in drug benefit management today. Furthermore, CMS oversight will ensure that Part D plans operate in accordance with this strategy. We will compare proposed Part D formularies using current best practices for developing and maintaining a formulary's drug classes and categories, and will support the use of USP model classes and categories for plans that choose to use them (plans are not required to do so). However, because drug classes alone, whether detailed or general, are not sufficient to determine adequate access for all beneficiaries, we also will review the drug plans' formularies and benefits to identify discriminating practices. Under Section 1860D-11(e)(2)(D) of the MMA, a plan design will be approved only if "the Secretary does not find that the design of the plan and its benefits (including any formulary and tiered formulary structure) are likely to substantially discourage enrollment by certain part D eligible individuals under the plan." Thus, even if CMS concludes that a plan's therapeutic classes and categories are non-discriminatory, CMS may find the plan design discriminatory if some other aspect of the plan's benefit design is problematic.

For example, CMS has the discretion to find that failure to include a specific drug would substantially discourage enrollment by beneficiaries with a condition that may only be treated by that drug. CMS is looking to existing formulary practices and national standards to inform its review at the specific drug level, and plans would be expected to accommodate widely applied guidelines and support current treatment options for conditions such as asthma, diabetes, behavioral health and psychological disorders, lipid disorders, hypertension and HIV.

CMS intends to encourage and approve formularies that provide the types of drug lists and benefit management approaches that are already in widespread use to provide drug coverage to millions of seniors and people with disabilities today. In addition to determining that the categories and classes and the formulary list offered are not discriminatory, CMS intends to check the plan design, using clear benchmarks that plans can utilize as a guide in building formularies and structuring their bids. We will consider the structure and use of an organization's P&T committee, as well as the structure of the formulary and the policies and procedures for providing access to both formulary and non-formulary drugs. Since drug utilization management activities are as important as the list of drugs in the formulary in providing access to high quality pharmaceutical care for all categories of beneficiaries, we will use benchmarks based on commonly-used best practices to review those policies and procedures as well as to ensure beneficiary access to Part D covered prescription drugs that are medically necessary for their course of treatment.

The extensive private sector experience with best practices involving P&T committees and other aspects of formulary oversight gives CMS a range of benchmarks that could be used to confirm that a formulary reflects current effective approaches to providing drug coverage. CMS intends to look to existing national standards and guidelines such as those established by URAC, the National Committee for Quality Assurance (NCQA), the American Society of Health System Pharmacists (ASHP) and the Academy of Managed Care Pharmacy (AMCP) to develop a framework for formulary management. The principles embodied in these standards and guidelines represent best practices that have been used widely and successfully, and we believe beneficiaries can gain from the application of these same widely used approaches in Medicare prescription drug benefit plans.

This strategy paper does not interfere with or supercede our review of public comments received under the proposed rule for the Medicare drug program; rather the details provided in this strategy paper are intended to help elicit public input on the specific approach we will use to conduct the formulary review under the final regulations. Modification to the regulation based on public comment may require some changes to this proposed strategy.

3. Guiding Principles for CMS Formulary and Benefit Review Strategy

A formulary is more than a list of approved medications. A formulary must consist of drugs that will provide patients with a clinically appropriate medication for the course of treatment established by the physician. Consistent with industry standards/practices, the formulary is supported by a system of care management tools to consistently provide patients with access to medications that have been demonstrated to be safe, effective, and affordable, while maintaining and improving quality patient care. To ensure that Medicare prescription drug plans are following best practices, the CMS formulary review will follow four important principles.

Principle #1 – Rely On Existing Best Practices. CMS' review will rely on widely recognized best practices for existing drug benefits serving millions of seniors and people with disabilities, to ensure non-discriminating, appropriate access for Medicare beneficiaries.

Principle #2 -- Provide Access to Medically Necessary Drugs: We will require that drug plans provide access to Part D drugs determined to be medically necessary and do not discriminate against any particular types of beneficiaries.

Principle #3 -- Flexibility: CMS will allow plans to be flexible in their benefit designs to promote real beneficiary choice while protecting beneficiaries from discrimination.

Principle #4 – Administrative Efficiency: CMS will develop a streamlined process to conduct effective reviews of plan offerings within a compressed period of time.

4. Strategic Approach

A. **P & T Committee Review**

We believe that current best practices for P&T committees should be applied when developing and administering P&T committees for the Medicare drug benefit. Incorporating best practice philosophies, along with inclusion of the MMA requirements, allows for a drug benefit that is both clinically sound and nondiscriminatory.

Rationale

The P&T committee is a vital pharmacy benefit best practice and a requirement of the MMA. Operated under appropriate guiding principles, a P&T committee is a forum for an evidence-based formulary review process that establishes policies on the use of drug products and therapies, and identifies drug products and therapies that are medically appropriate and cost-effective. Reflecting MMA requirements, P&T committees must meet best practices consistent with those contained in several widely accepted guidelines (and many lesser recognized guidelines) for P&T management. CMS standards and guidelines for the P&T activities will help ensure that formulary decisions are based on scientific and economic considerations that achieve appropriate, safe and cost effective drug therapy, and that the P&T committee has a key role in defining policies for

utilization management activities such as access to non-formulary drugs, prior authorization, step therapy, generic substitution, and therapeutic interchange protocols to assure that products and therapies, such that these tools are used to drive medically appropriate and cost-effective access to Part D covered drugs. The P&T committee will also be expected to analyze and recommend, where appropriate, regional variations of national best practices.

These standards will be clearly articulated in the plan applications and our contracts with PDPs. They will also be integrated into the CMS management and oversight of Part D plans after January 2006, to assure that the P&T rules are maintained and followed.

Approach

CMS will require that plans guarantee the implementation and use of a P&T committee consistent with the pharmacy benefit management principles outlined and expressed by the American Society of Health System Pharmacists (ASHP Statement on the Pharmacy and Therapeutics Committee, Am J Hosp Pharm. 1992, http://www.ashp.org/), or the Academy of Managed Care Pharmacy (AMCP Principles of a Sound Drug Formulary System October 2000, www.amcp.org).

The requirements listed below are represented as 'BP' for best practice (or Industry Standard Practice) where they have been drawn from commercial best practice consistent with these nationally recognized P&T guidelines, and are represented as 'MMA' where the requirements support the unique provisions of the MMA.

Membership

- P&T committee members must represent various clinical specialties that adequately represent the needs of plans beneficiaries (i.e., include representation of "high volume specialists" in the standard terminology of the industry). (BP)

- A majority of the P&T committee members must be practicing physicians, practicing pharmacists or both. (BP)

- At least one P&T committee practicing pharmacist and one practicing physician must be experts in the care of elderly or disabled persons. (MMA)

- At least one P&T committee practicing pharmacist and one practicing physician must be independent and free of conflict with respect to the sponsor and plan. (MMA)

Conflict of Interest

- P&T committee members should sign a conflict of interest statement revealing economic or other relationships with entities affected by drug coverage decisions that could influence committee decisions. (BP)

Meeting Administration

- P&T committee should meet on a regular basis, and not less frequently than on a quarterly basis. (BP)

- P&T committee decisions regarding formulary development or revision must be documented in writing. (BP)

Formulary Management

- P&T committee must review for clinical appropriateness, the practices and policies for formulary management activities, such as prior authorizations, step therapies, generic substitutions and other drug utilization activities that affect access. (BP)

- Formulary management decisions must be based on scientific evidence, and may also be based on pharmacoeconomic considerations that achieve appropriate, safe and cost effective drug therapy. (BP)

- The P&T committees will be required to establish procedures to assure appropriate drug review and inclusion. (BP)

- Clinical decisions by the P&T committee should be based on scientific evidence and standards of practice, including peer reviewed medical literature, well-established clinical practice guidelines and pharmacoeconomic studies as well as other sources of appropriate information. (BP)

- Drugs' therapeutic advantages in terms of safety and efficacy must be considered when selecting formulary drugs and placing them into formulary tiers. (MMA)

- The P&T committee must review each new chemical entity within 90 days of its release onto the market, or a clinical justification must be provided if this timeframe is not met. (BP)

- P&T committee will approve inclusion or exclusion of the therapeutic classes in the formulary on an annual basis. (MMA)

- Formulary therapeutic categories and classes may be changed only at the beginning of each plan year or when new drugs or new drug therapeutic uses appear. (MMA)

Formulary Exceptions

- P&T committees must establish protocols and procedures for the timely use of and access to both formulary and non-formulary drug products. A non-formulary drug may be needed, for example, when the formulary drug would cause adverse effects or would not be effective or both, based on scientific evidence or medical necessity. (BP)

B. Formulary List Review

Rationale

The formulary list review will incorporate best practices from the private sector, Medicaid and FEHB formularies. The MMA requires CMS to review Part D formularies to ensure that beneficiaries have access to a broad range of medically appropriate drugs to treat all disease states and to ensure that the formulary design does not discriminate or substantially discourage enrollment by certain groups. We expect that the kinds of formularies in widespread use today, which provide high-quality drug coverage to millions of Medicare beneficiaries, would receive a straightforward approval under this approach (with modifications to account for specific features of Medicare's benefit structure, e.g., including home infusion products that might be covered under a medical benefit in commercial benefit designs). Below we provide a preliminary series of checks that CMS proposes to use to confirm that plan formularies will provide the kind of effective, non-discriminatory access available in drug benefit plans today. The final guidelines will be released in January, based on public comments to this document.

Approach

We encourage plans to submit formularies similar to those in widespread use today. We will check the formulary to ensure inclusion of a range of drugs in a broad distribution of therapeutic categories and classes, to satisfy the MMA requirement that categorization systems do not discourage enrollment to any group of beneficiaries. Additionally, we will check the formulary against a range of reasonable benchmarks to confirm that formularies are consistent with best practice. We also will consider the specific drugs, tiering and utilization management strategies employed in each formulary. Outliers will be identified for further review. CMS will ask for clinical justification from plans that differ from these features.

Review of Categories and Classes.

We will review all classification systems to assure that plans provide an appropriate breadth of categories and classes that cover all disease states. Plans can choose to adopt a classification system that is consistent with the USP's drug categories and classes or may use other classification systems. CMS will not consider a classification system in isolation from the subsequent steps in our

formulary review; a system with a smaller number of classes may be acceptable if it nonetheless provides preferred access to a relatively broad range of widely used medicines.

Plans using the USP classification system will satisfy a safe harbor and thus CMS will approve their formulary classification system. We note that the MMA says that categorization systems that are "consistent with" the model classification system will get the safe harbor. While it is obvious that plans that adopt the USP system in its entirety would have access to the safe harbor, CMS requests comments on what other classification systems should have this safe harbor based on being "consistent with" the USP classification system. The number and specific choices of drugs must still pass CMS's drug list review (see below). As a general matter, these drugs are not required to be on any particular tier of the plan's formulary (generic, preferred brand, non-preferred brand, etc.), but we will review the tier placement to ensure that it does not discourage enrollment by any specific group of beneficiaries.

For plans that choose to adopt an alternative to the USP's structure, CMS will check the plan's proposed classification system to determine if it is similar to other commonly used classification systems, such as American Hospital Formulary System (AHFS) or those used in popular FEHB plans and other plans that serve many seniors and persons with disabilities. Again the number of and specific drugs chosen must still pass CMS's drug list review (see below). CMS seeks comments on which existing classification systems could provide appropriate standards, keeping in mind that CMS does not view the classification system alone as the principal determinant of formulary adequacy.

Plan sponsors will also have the opportunity to demonstrate to CMS that their proposed classification system is in use to provide drug benefits to a significant number of Medicare beneficiaries. If we find that the proposed classification system is in use for many beneficiaries, we will approve the classification system.

Drug List Review

Regardless of the classification system chosen, CMS will look to approve drug lists that are consistent with formularies currently in widespread use.
CMS may use a variety of benchmarks to confirm that the specific drugs in each category and class provide a sufficient breadth of drugs necessary to treat all disease states in a non-discriminatory way. The minimum statutory requirement is that the formulary includes at least two drugs in each approved category and class. We view this requirement as a floor rather than an absolute standard. CMS may require more than two drugs per class in cases where additional drugs present unique and important therapeutic advantages in terms of safety and efficacy. Also, inclusion of drugs in a formulary does not by itself imply that the formulary is adequate. Rather, we will review tier placement (generic, preferred brand, non preferred brand, etc), to provide an assurance that the formulary is non-

discriminatory. It is our understanding that best practices in existing formularies and Medicaid preferred drug lists generally place drugs in a less preferable position only when drugs that are therapeutically equivalent (i.e., drugs that provide similar treatment outcomes) are in more preferable positions on the formulary.

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To conduct these reviews, CMS will benchmark each proposed formulary against existing widely-used formularies and Medicaid preferred drug lists that provide broad coverage for seniors and persons with disabilities, and against other proposed Part D formularies, to identify outliers in formulary and pharmacy benefit management best practices.

CMS is considering a number of potential benchmarks to confirm the adequacy of a formulary drug list. As one possible benchmark, CMS would analyze the availability and tier position of the commonly prescribed drugs, particularly the top 25-50 drugs for the Medicare population in terms of cost and utilization. CMS understands that plans will not provide identical coverage of these drugs, and our review will focus on assuring that plans present a balanced formulary. These drugs will cover common diseases and conditions, and will allow us to ensure that plans are covering the most widely used medications or therapeutic equivalents for the most common conditions. CMS will also assess the availability and tier position for commonly prescribed drugs for uncommon conditions.

CMS will also consider widely accepted treatment guidelines. CMS will analyze whether appropriate access is afforded to drugs addressed in widely accepted treatment guidelines, such as those for asthma, diabetes, behavioral health and psychological disorders, lipid disorders, hypertension, HIV/AIDS, etc. It is our understanding that the inclusion of these drugs or drug classes will not place undue burden on plans since these drugs are usually placed in favorable positions on commonly used, best practice formularies. In some cases, widespread industry practices and widely used treatment guidelines require all or substantially all drugs in a particular class to be covered. CMS will identify the drugs and treatment guidelines where favorable coverage is considered based on such common industry best practices, and when these drugs are only available in non-preferred positions, CMS will review the plan's justification for the placement. CMS will also use Part D risk adjustment data to analyze proposed formularies at the drug level to ensure that the most commonly covered drugs for the Medicare population are represented across disease categories. CMS seeks comments as to the types of treatment guidelines that should be considered in our review.

CMS is also considering the use of a check of whether the formulary includes at least one drug from each of USP's recommended categories and classes and sub-divisions (column 3 in USP's proposed classification system) in our review of drug lists as a proxy for comprehensiveness. We encourage comment as to the appropriateness of this tool.

Examples of outliers identified in the above processes are: failure to compare favorably to the commonly used, comprehensive drug classification systems noted above, exclusion of all drugs from drug classes that are commonly used, and the placement of drugs in non-preferred tiers in the absence of commonly used therapeutic equivalents on more preferred positions. Plans will have the flexibility to clinically justify deviations when formulary lists vary from widely used best practices.

CMS will monitor changes to approved formularies on an ongoing basis and initiate discussion when necessary to assure that a formulary remains non-discriminatory.

C. Review of Benefit Management Tools that Affect Access

Rationale

CMS will review plans' use of utilization management tools, including prior authorization, step therapy and generic substitution to ensure that beneficiaries are given appropriate access to drugs in a timely manner. We will also review plans' drug utilization review procedures and appeals, exceptions and grievances processes. Our review will focus on ensuring that all of these plan systems reflect current best practices that are utilized in the private sector, Medicaid and FEHB plans.

Approach

Prior Authorization, Step Therapy, Generic Substitution

CMS will look to existing best practices to check that plans' use of these utilization management tools is consistent with such practices. We will look to current industry standards as well as appropriate guidelines that might be found from expert organizations such as NCQA, AMCP, and NAIC, and to the use of such standards in existing drug plans that are widely used by seniors and people with disabilities. CMS will assure that plans' use of such tools do not create a discriminatory environment. CMS will also analyze and benchmark proposed formularies in comparison to formularies and preferred drug lists widely used by Medicare beneficiaries on the comparative use of practices such as prior authorization, step therapy, and quantity limits. Our expectation is that these techniques will be used in Part D formularies consistently with the way they are applied in existing formulary systems, both in terms of the situations in which they are used and the timeliness of the processes.

Drug Utilization Review (DUR)

CMS will review plans' DUR practices to confirm that they meet industry best practices in terms of access to drugs and quality oversight. We will expect plans'

use of tools and techniques currently in place in their commercial coverage business. These processes may include concurrent review as well as prospective and/or retrospective utilization review. These reviews will be expected to assure appropriate access to medically necessary therapies as well as guard against inappropriate or dangerous utilization of prescription medications.

Appeals, Exceptions and Grievances

As part of our work to finalize the drug benefit regulation, CMS is considering numerous comments related to appeals, exceptions and grievances. The standards for handling appeals, exceptions, and grievances will be specific and contained in our final rule, not sub regulatory guidance. Our expectation is that the final rule will reflect current best practices around appeal and grievance timeframes and we are developing notice requirements to ensure that beneficiaries understand their rights in this area. We also expect to require standardized reporting from Part D plans on denial, reconsideration and appeals, and exceptions processing and integrate this data into CMS management and oversight activities. This will assure that plans make appropriate use of the data for internal quality initiatives, such as those directed at managing excessive rates of overturned utilization management decisions.

5. Next Steps

We have begun reviewing national standards and working with contractors to operationalize our requirements. In the interest of plans being successful in submitting applications and bids that meet our requirements, CMS intends to finalize this work in early 2005, well in advance of bids being submitted. Importantly, this approach will also serve to assure beneficiaries and other parties that the benefit packages are structured in accordance with our requirements and that they are nondiscriminatory. CMS invites public comment on our proposed strategy. Final formulary guidelines will be published early in 2005 after receipt of public comments from this draft and after publication of the final rule.